Summary conclusions from the GMP workshop

The GMP workshop was extremely useful for identifying areas where CIRM can play a critical role for insuring that California develops and maintains sufficient GMP facilities to support CIRM awardees as they move their cell therapy research discoveries to clinical testing in patients. There is growing capacity for cell production under GMP conditions in the State, both in the public and private sectors, but there are important areas of unmet needs in terms of personnel, research and access. CIRM can assist in each of these areas.

Personnel - Most participants agreed that finding trained technicians to operate GMP facilities is difficult, especially for projects related to cell therapy. The number of trained individuals is currently insufficient and training new technicians is a long process, taking up to two years. To help address this problem, CIRM should develop an RFA over the next two years for training grants similar to the Bridges program, but targeted to training students for positions in GMP facilities. These programs should include extensive technical training, including internships within GMP facilities either at universities or companies. Such programs cannot be expected to fully train individuals because different facilities currently employ different procedures and protocols. However, they will introduce a new cadre of students to career opportunities in this expanding industry and shorten the training period once graduates are employed.

Research – There was consensus in the group that conditions for isolating, expanding, maintaining and banking stem cells under GMP conditions have not been optimized or standardized. Research is needed in this area, if the field is to progress to phase 3 human trials, and funding for such research has been lacking. To help fill this gap, CIRM should address the need for research in these areas in the next two years by providing grants (and possibly loans) for research aimed at optimizing methods for deriving, maintaining and expanding stem cells under GMP (including xeno-free) conditions. (NOTE: GMP scale-up is a bottleneck for translation, and so research in this area can be made a priority in an Early Translation core grant.) Progress in this area will benefit all research programs focused on stem cell therapies in humans.

Access – As research findings move from the laboratory to human trials, an increasing number of CIRM-funded investigators will require access to stem cells derived, maintained and differentiated under GMP conditions. To meet this need, CIRM should seek mechanisms to insure adequate supplies. This effort could include RFPs designed to attract organizations or consortia capable of deriving and maintaining stem cells lines under GMP conditions for use by CIRM grantees.

High quality GMP facilities will be essential in the development of cell therapies for human trials. Universities and companies have begun to gear up to meet this growing need. However, such facilities are expensive to operate and difficult to staff. By developing targeted programs for training, research and support, CIRM can help insure that appropriate facilities will be available for its awardees as they move stem cell biology toward human therapeutics.